IFWO

```
RAW SEQUENCE LISTING                    DATE: 08/31/2004
PATENT APPLICATION:  US/10/722,371      TIME: 13:37:50

        Input Set : N:\Crf3\RULE60\10722371.raw.txt
        Output Set: N:\CRF4\08312004\J722371.raw
```
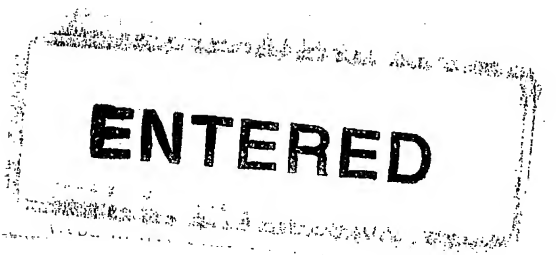
```
 1 <110> APPLICANT: KAKKIS, EMIL D.
 2 <120> TITLE OF INVENTION: METHODS FOR TREATING DISEASES CAUSED BY DEFICIENCIES OF
 3       RECOMBINANT ALPHA-L-IDURONDINASE
 4 <130> FILE REFERENCE: 008000051CNUS01
 5 <140> CURRENT APPLICATION NUMBER: US/10/722,371
 6 <141> CURRENT FILING DATE: 2003-11-24
 7 <150> PRIOR APPLICATION NUMBER: US/09/993,038
 8 <151> PRIOR FILING DATE: 2001-11-13
 9 <150> PRIOR APPLICATION NUMBER: 09/711,205
10 <151> PRIOR FILING DATE: 2000-11-09
11 <150> PRIOR APPLICATION NUMBER: 09/439,923
12 <151> PRIOR FILING DATE: 1999-11-12
13 <160> NUMBER OF SEQ ID NOS: 2
14 <170> SOFTWARE: FastSEQ for Windows Version 3.0
16 <210> SEQ ID NO: 1
17 <211> LENGTH: 6200
18 <212> TYPE: DNA
19 <213> ORGANISM: Homo sapiens
20 <220> FEATURE:
21 <221> NAME/KEY: CDS
22 <222> LOCATION: (1558)...(3510)
23 <400> SEQUENCE: 1
24       gacggatcgg gagatctccc gatcccctat ggtcgactct cagtacaatc tgctctgatg      60
25       ccgcatagtt aagccagtat ctgctccctg cttgtgtgtt ggaggtcgct gagtagtgcg     120
26       cgagcaaaat ttaagctaca acaaggcaag gcttgaccga caattgcatg aagaatctgc     180
27       ttagggttag gcgttttgcg ctgcttcgcg atgtacgggc cagatatacg cgttgacatt     240
28       gattattgac tagttattaa tagtaatcaa ttacggggtc attagttcat agcccatata     300
29       tggagttccg cgttacataa cttacggtaa atggcccgcc tggctgaccg cccaacgacc     360
30       cccgcccatt gacgtcaata atgacgtatg ttcccatagt aacgccaata gggactttcc     420
31       attgacgtca atgggtggac tatttacggt aaactgccca cttggcagta catcaagtgt     480
32       atcatatgcc aagtacgccc cctattgacg tcaatgacgg taaatggccc gcctggcatt     540
33       atgcccagta catgacctta tgggactttc ctacttggca gtacatctac gtattagtca     600
34       tcgctattac catggtgatg cggttttggc agtacatcaa tgggcgtgga tagcggtttg     660
35       actcacgggg atttccaagt ctccacccca ttgacgtcaa tgggagtttg ttttggcacc     720
36       aaaatcaacg ggactttcca aaatgtcgta acaactccgc cccattgacg caaatgggcg     780
37       gtaggcgtgt acggtgggag gtctatataa gcagagctct ctggctaact agagaaccca     840
38       ctgcttaact ggcttatcga aattaatacg actcactata gggagaccca agcttcgcag     900
39       aattcctgcg gctgctacag tgtgtccagc gtcctgcctg gctgtgctga gcgctggaac     960
40       agtggcgcat cattcaagtg cacagttacc catcctgagt ctggcacctt aactggcaca    1020
41       attgccaaag tcacaggtga gctcagatgc ataccaggac attgtatgac gttccctgct    1080
42       cacatgcctg ctttcttcct ataatacaga tgctcaacta actgctcatg tccttatatc    1140
43       acagagggaa attggagcta tctgaggaac tgcccagaag ggaagggcag aggggtcttg    1200
44       ctctccttgt ctgagccata actcttcttt ctaccttcca gtgaacacct cccacccca     1260
```

```
45   ggtccacctg ctaccgccgc cgtcggagga gctggccctg aatgagctct tgtccctgac   1320
46   atgcctggtg cgagctttca accctaaaga agtgctggtg cgatggctgc atggaaatga   1380
47   ggagctgtcc ccagaaagct acctagtgtt tgagccccta aaggagccag gcgagggagc   1440
48   caccacctac ctggtgacaa gcgtgttgcg tgtatcagct gaaagcttga tatcgaattc   1500
49   cggaggcgga accggcagtg cagcccgaag ccccgcagtc cccgagcacg cgtggcc atg   1560
50                                                                   Met
51                                                                     1

52   cgt ccc ctg cgc ccc cgc gcc gcg ctg ctg gcg ctc ctg gcc tcg ctc   1608
53   Arg Pro Leu Arg Pro Arg Ala Ala Leu Leu Ala Leu Leu Ala Ser Leu
54                   5                  10                  15

55   ctg gcc gcg ccc ccg gtg gcc ccg gcc gag gcc ccg cac ctg gtg cat   1656
56   Leu Ala Ala Pro Pro Val Ala Pro Ala Glu Ala Pro His Leu Val His
57               20                  25                  30

58   gtg gac gcg gcc cgc gcg ctg tgg ccc ctg cgg cgc ttc tgg agg agc   1704
59   Val Asp Ala Ala Arg Ala Leu Trp Pro Leu Arg Arg Phe Trp Arg Ser
60           35                  40                  45

61   aca ggc ttc tgc ccc ccg ctg cca cac agc cag gct gac cag tac gtg   1752
62   Thr Gly Phe Cys Pro Pro Leu Pro His Ser Gln Ala Asp Gln Tyr Val
63   50                  55                  60                  65

64   ctc agc tgg gac cag cag ctc aac ctc gcc tat gtg ggc gcc gtc cct   1800
65   Leu Ser Trp Asp Gln Gln Leu Asn Leu Ala Tyr Val Gly Ala Val Pro
66                   70                  75                  80

67   cac cgc ggc atc aag cag gtc cgg acc cac tgg ctg ctg gag ctt gtc   1848
68   His Arg Gly Ile Lys Gln Val Arg Thr His Trp Leu Leu Glu Leu Val
69               85                  90                  95

70   acc acc agg ggg tcc act gga cgg ggc ctg agc tac aac ttc acc cac   1896
71   Thr Thr Arg Gly Ser Thr Gly Arg Gly Leu Ser Tyr Asn Phe Thr His
72               100                 105                 110

73   ctg gac ggg tac ctg gac ctt ctc agg gag aac cag ctc ggg ttt gag   1944
74   Leu Asp Gly Tyr Leu Asp Leu Leu Arg Glu Asn Gln Leu Gly Phe Glu
75           115                 120                 125

76   ctg atg ggc agc gcc tcg ggc cac ttc act gac ttt gag gac aag cag   1992
77   Leu Met Gly Ser Ala Ser Gly His Phe Thr Asp Phe Glu Asp Lys Gln
78   130                 135                 140                 145

79   cag gtg ttt gag tgg aag gac ttg gtc tcc agc ctg gcc agg aga tac   2040
80   Gln Val Phe Glu Trp Lys Asp Leu Val Ser Ser Leu Ala Arg Arg Tyr
81                   150                 155                 160

82   atc ggt agg tac gga ctg gcg cat gtt tcc aag tgg aac ttc gag acg   2088
83   Ile Gly Arg Tyr Gly Leu Ala His Val Ser Lys Trp Asn Phe Glu Thr
84               165                 170                 175

85   tgg aat gag cca gac cac cac gac ttt gac aac gtc tcc atg acc atg   2136
86   Trp Asn Glu Pro Asp His His Asp Phe Asp Asn Val Ser Met Thr Met
87               180                 185                 190

88   caa ggc ttc ctg aac tac tac gat gcc tgc tcg gag ggt ctg cgc gcc   2184
89   Gln Gly Phe Leu Asn Tyr Tyr Asp Ala Cys Ser Glu Gly Leu Arg Ala
90               195                 200                 205

91   gcc agc ccc gcc ctg cgg ctg gga ggc ccc ggc gac tcc ttc cac acc   2232
92   Ala Ser Pro Ala Leu Arg Leu Gly Gly Pro Gly Asp Ser Phe His Thr
93   210                 215                 220                 225
```

```
94    cca ccg cga tcc ccg ctg agc tgg ggc ctc ctg cgc cac tgc cac gac    2280
95    Pro Pro Arg Ser Pro Leu Ser Trp Gly Leu Leu Arg His Cys His Asp
96                    230                 235                 240
97    ggt acc aac ttc ttc act ggg gag gcg ggc gtg cgg ctg gac tac atc    2328
98    Gly Thr Asn Phe Phe Thr Gly Glu Ala Gly Val Arg Leu Asp Tyr Ile
99                    245                 250                 255
100   tcc ctc cac agg aag ggt gcg cgc agc tcc atc tcc atc ctg gag cag    2376
101   Ser Leu His Arg Lys Gly Ala Arg Ser Ser Ile Ser Ile Leu Glu Gln
102                   260                 265                 270
103   gag aag gtc gtc gcg cag cag atc cgg cag ctc ttc ccc aag ttc gcg    2424
104   Glu Lys Val Val Ala Gln Gln Ile Arg Gln Leu Phe Pro Lys Phe Ala
105               275                 280                 285
106   gac acc ccc att tac aac gac gag gcg gac ccg ctg gtg ggc tgg tcc    2472
107   Asp Thr Pro Ile Tyr Asn Asp Glu Ala Asp Pro Leu Val Gly Trp Ser
108   290                 295                 300                 305
109   ctg cca cag ccg tgg agg gcg gac gtg acc tac gcg gcc atg gtg gtg    2520
110   Leu Pro Gln Pro Trp Arg Ala Asp Val Thr Tyr Ala Ala Met Val Val
111                   310                 315                 320
112   aag gtc atc gcg cag cat cag aac ctg cta ctg gcc aac acc acc tcc    2568
113   Lys Val Ile Ala Gln His Gln Asn Leu Leu Leu Ala Asn Thr Thr Ser
114                   325                 330                 335
115   gcc ttc ccc tac gcg ctc ctg agc aac gac aat gcc ttc ctg agc tac    2616
116   Ala Phe Pro Tyr Ala Leu Leu Ser Asn Asp Asn Ala Phe Leu Ser Tyr
117                   340                 345                 350
118   cac ccg cac ccc ttc gcg cag cgc acg ctc acc gcg cgc ttc cag gtc    2664
119   His Pro His Pro Phe Ala Gln Arg Thr Leu Thr Ala Arg Phe Gln Val
120           355                 360                 365
121   aac aac acc cgc ccg ccg cac gtg cag ctg ttg cgc aag ccg gtg ctc    2712
122   Asn Asn Thr Arg Pro Pro His Val Gln Leu Leu Arg Lys Pro Val Leu
123   370                 375                 380                 385
124   acg gcc atg ggg ctg ctg gcg ctg ctg gat gag gag cag ctc tgg gcc    2760
125   Thr Ala Met Gly Leu Leu Ala Leu Leu Asp Glu Glu Gln Leu Trp Ala
126                   390                 395                 400
127   gaa gtg tcg cag gcc ggg acc gtc ctg gac agc aac cac acg gtg ggc    2808
128   Glu Val Ser Gln Ala Gly Thr Val Leu Asp Ser Asn His Thr Val Gly
129               405                 410                 415
130   gtc ctg gcc agc gcc cac cgc ccc cag ggc ccg gcc gac gcc tgg cgc    2856
131   Val Leu Ala Ser Ala His Arg Pro Gln Gly Pro Ala Asp Ala Trp Arg
132               420                 425                 430
133   gcc gcg gtg ctg atc tac gcg agc gac gac acc cgc gcc cac ccc aac    2904
134   Ala Ala Val Leu Ile Tyr Ala Ser Asp Asp Thr Arg Ala His Pro Asn
135           435                 440                 445
136   cgc agc gtc gcg gtg acc ctg cgg ctg cgc ggg gtg ccc ccc ggc ccg    2952
137   Arg Ser Val Ala Val Thr Leu Arg Leu Arg Gly Val Pro Pro Gly Pro
138   450                 455                 460                 465
139   ggc ctg gtc tac gtc acg cgc tac ctg gac aac ggg ctc tgc agc ccc    3000
140   Gly Leu Val Tyr Val Thr Arg Tyr Leu Asp Asn Gly Leu Cys Ser Pro
141                   470                 475                 480
142   gac ggc gag tgg cgg cgc ctg ggc cgg ccc gtc ttc ccc acg gca gag    3048
```

```
143     Asp Gly Glu Trp Arg Arg Leu Gly Arg Pro Val Phe Pro Thr Ala Glu
144                 485             490             495
145     cag ttc cgg cgc tag cgc gcg gct gag gac ccg gtg gcc gcg gcg ccc    3096
146     Gln Phe Arg Arg  *  Arg Ala Ala Glu Asp Pro Val Ala Ala Ala Pro
147             500             505             510
148     cgc ccc tta ccc gcc ggc ggc cgc ctg agg ctg cgc ccc gcg ctg cgg    3144
149     Arg Pro Leu Pro Ala Gly Gly Arg Leu Arg Leu Arg Pro Ala Leu Arg
150             515             520             525
151     ctg ccg tcg ctt ttg ctg gtg cac gtg tgt gcg cgc ccc gag aag ccg    3192
152     Leu Pro Ser Leu Leu Leu Val His Val Cys Ala Arg Pro Glu Lys Pro
153             530             535             540
154     ccc ggg cag gtc acg cgg ctc cgc gcc ctg ccc ctg acc caa ggg cag    3240
155     Pro Gly Gln Val Thr Arg Leu Arg Ala Leu Pro Leu Thr Gln Gly Gln
156     545             550             555             560
157     ctg gtt ctg gtc tgg tcg gat gaa cac gtg ggc tcc aag tgc ctg tgg    3288
158     Leu Val Leu Val Trp Ser Asp Glu His Val Gly Ser Lys Cys Leu Trp
159                 565             570             575
160     aca tac gag atc cag ttc tct cag gac ggt aag gcg tac acc ccg gtc    3336
161     Thr Tyr Glu Ile Gln Phe Ser Gln Asp Gly Lys Ala Tyr Thr Pro Val
162                 580             585             590
163     agc agg aag cca tcg acc ttc aac ctc ttt gtg ttc agc cca gac aca    3384
164     Ser Arg Lys Pro Ser Thr Phe Asn Leu Phe Val Phe Ser Pro Asp Thr
165                 595             600             605
166     ggt gct gtc tct ggc tcc tac cga gtt cga gcc ctg gac tac tgg gcc    3432
167     Gly Ala Val Ser Gly Ser Tyr Arg Val Arg Ala Leu Asp Tyr Trp Ala
168             610             615             620
169     cga cca ggc ccc ttc tcg gac cct gtg ccg tac ctg gag gtc cct gtg    3480
170     Arg Pro Gly Pro Phe Ser Asp Pro Val Pro Tyr Leu Glu Val Pro Val
171     625             630             635             640
172     cca aga ggg ccc cca tcc ccg ggc aat cca tgagcctgtg ctgagcccca    3530
173     Pro Arg Gly Pro Pro Ser Pro Gly Asn Pro
174                 645             650
175     gtgggttgca cctccaccgg cagtcagcga gctggggctg cactgtgccc atgctgccct    3590
176     cccatcaccc cctttgcaat atattttat attttaaaaa aaaaaaaaa aaaaaaaaa    3650
177     aaaaaaaaaa aaaaaaaaaa aaaaaaaaaa aaaaaaaaaa aaaaaaaaag aattcctgca    3710
178     gcccggggga tccactagtt ctagagggcc cgtttaaacc cgctgatcag cctcgactgt    3770
179     gccttctagt tgccagccat ctgttgtttg ccctcccccc gtgccttcct tgaccctgga    3830
180     aggtgccact cccactgtcc tttcctaata aaatgaggaa attgcatcgc attgtctgag    3890
181     taggtgtcat tctattctgg ggggtggggt ggggcaggac agcaaggggg aggattggga    3950
182     agacaatagc aggcatgctg gggatgcggt gggctctatg gcttctgagg cggaaagaac    4010
183     cagctggggc tcgagagctt ggcgtaatca tggtcatagc tgtttcctgt gtgaaattgt    4070
184     tatccgctca caattccaca caacatacga gccggaagca taaagtgtaa agcctggggt    4130
185     gcctaatgag tgagctaact cacattaatt gcgttgcgct cactgcccgc tttccagtcg    4190
186     ggaaacctgt cgtgccagct gcattaatga atcggccaac gcgcggggag aggcggtttg    4250
187     cgtattgggc gctcttccgc ttcctcgctc actgactcgc tgcgctcggt cgttcggctg    4310
188     cggcgagcgg tatcagctca ctcaaaggcg gtaatacggt tatccacaga atcaggggat    4370
189     aacgcaggaa agaacatgtg agcaaaaggc cagcaaaagg ccaggaaccg taaaaaggcc    4430
190     gcgttgctgg cgtttttcca taggctccgc cccccctgacg agcatcacaa aaatcgacgc    4490
191     tcaagtcaga ggtggcgaaa cccgacagga ctataaagat accaggcgtt ccccctgga    4550
```

RAW SEQUENCE LISTING   DATE: 08/31/2004
PATENT APPLICATION: US/10/722,371   TIME: 13:37:50

        Input Set : N:\Crf3\RULE60\10722371.raw.txt
        Output Set: N:\CRF4\08312004\J722371.raw

```
192      agctccctcg tgcgctctcc tgttccgacc ctgccgctta ccggatacct gtccgccttt   4610
193      ctcccttcgg gaagcgtggc gctttctcaa tgctcacgct gtaggtatct cagttcggtg   4670
194      taggtcgttc gctccaagct gggctgtgtg cacgaacccc ccgttcagcc cgaccgctgc   4730
195      gccttatccg gtaactatcg tcttgagtcc aacccggtaa gacacgactt atcgccactg   4790
196      gcagcagcca ctggtaacag gattagcaga gcgaggtatg taggcggtgc tacagagttc   4850
197      ttgaagtggt ggcctaacta cggctacact agaaggacag tatttggtat ctgcgctctg   4910
198      ctgaagccag ttaccttcgg aaaaagagtt ggtagctctt gatccggcaa acaaaccacc   4970
199      gctggtagcg gtggtttttt tgtttgcaag cagcagatta cgcgcagaaa aaaggatct   5030
200      caagaagatc ctttgatctt ttctacgggg tctgacgctc agtggaacga aaactcacgt   5090
201      taagggattt tggtcatgag attatcaaaa aggatcttca cctagatcct tttaaattaa   5150
202      aaatgaagtt ttaaatcaat ctaaagtata tatgagtaaa cttggtctga cagttaccaa   5210
203      tgcttaatca gtgaggcacc tatctcagcg atctgtctat ttcgttcatc catagttgcc   5270
204      tgactccccg tcgtgtagat aactacgata cgggagggct taccatctgg ccccagtgct   5330
205      gcaatgatac cgcgagaccc acgctcaccg gctccagatt tatcagcaat aaaccagcca   5390
206      gccggaaggg ccgagcgcag aagtggtcct gcaactttat ccgcctccat ccagtctatt   5450
207      aattgttgcc gggaagctag agtaagtagt tcgccagtta atagtttgcg caacgttgtt   5510
208      gccattgcta caggcatcgt ggtgtcacgc tcgtcgtttg gtatggcttc attcagctcc   5570
209      ggttcccaac gatcaaggcg agttacatga tcccccatgt tgtgcaaaaa agcggttagc   5630
210      tccttcggtc ctccgatcgt tgtcagaagt aagttggccg cagtgttatc actcatggtt   5690
211      atggcagcac tgcataattc tcttactgtc atgccatccg taagatgctt ttctgtgact   5750
212      ggtgagtact caaccaagtc attctgagaa tagtgtatgc ggcgaccgag ttgctcttgc   5810
213      ccggcgtcaa tacgggataa taccgcgcca catagcagaa ctttaaaagt gctcatcatt   5870
214      ggaaaacgtt cttcggggcg aaaactctca aggatcttac cgctgttgag atccagttcg   5930
215      atgtaaccca ctcgtgcacc caactgatct tcagcatctt ttactttcac cagcgtttct   5990
216      gggtgagcaa aaacaggaag gcaaaatgcc gcaaaaaagg gaataagggc gacacggaaa   6050
217      tgttgaatac tcatactctt cctttttcaa tattattgaa gcatttatca gggttattgt   6110
218      ctcatgagcg gatacatatt tgaatgtatt tagaaaaata aacaaatagg ggttccgcgc   6170
219      acatttcccc gaaaagtgcc acctgacgtc                                    6200
221 <210> SEQ ID NO: 2
222 <211> LENGTH: 650
223 <212> TYPE: PRT
224 <213> ORGANISM: Homo sapiens
225 <400> SEQUENCE: 2
226      Met Arg Pro Leu Arg Pro Arg Ala Ala Leu Leu Ala Leu Leu Ala Ser
227       1               5                  10                  15
228      Leu Leu Ala Ala Pro Pro Val Ala Pro Ala Glu Ala Pro His Leu Val
229                      20                  25                  30
230      His Val Asp Ala Ala Arg Ala Leu Trp Pro Leu Arg Arg Phe Trp Arg
231                  35                  40                  45
232      Ser Thr Gly Phe Cys Pro Pro Leu Pro His Ser Gln Ala Asp Gln Tyr
233              50                  55                  60
234      Val Leu Ser Trp Asp Gln Gln Leu Asn Leu Ala Tyr Val Gly Ala Val
235      65                  70                  75                  80
236      Pro His Arg Gly Ile Lys Gln Val Arg Thr His Trp Leu Leu Glu Leu
237                      85                  90                  95
238      Val Thr Thr Arg Gly Ser Thr Gly Arg Gly Leu Ser Tyr Asn Phe Thr
239                      100                 105                 110
240      His Leu Asp Gly Tyr Leu Asp Leu Leu Arg Glu Asn Gln Leu Gly Phe
241                  115                 120                 125
```

RAW SEQUENCE LISTING ERROR SUMMARY   DATE: 08/31/2004
PATENT APPLICATION: **US/10/722,371**   TIME: 13:37:51

Input Set : **N:\Crf3\RULE60\10722371.raw.txt**
Output Set: **N:\CRF4\08312004\J722371.raw**

<u>Invalid Line Length:</u>
The rules require that a line not exceed 72 characters in length. This includes spaces.

Seq#:1; Line(s) 2,49

VERIFICATION SUMMARY
PATENT APPLICATION: **US/10/722,371**

DATE: 08/31/2004
TIME: 13:37:51

Input Set : **N:\Crf3\RULE60\10722371.raw.txt**
Output Set: **N:\CRF4\08312004\J722371.raw**